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Department of Health and Human Services (HHS)

Re: Medicare Prescription Drug, Improvement, and Modernization Act of 2003, Section 1013: Suggested Priority Topics for Research. Docket No. 2004S-0170: 69 Fed. Reg. 22045 (April 23, 2004).

The Pharmaceutical Research and Manufacturers of America ("PhRMA") is pleased to submit these recommendations for research priorities for the Agency for Healthcare Research and Quality (AHRQ) under Section 1013 of the Medicare Prescription Drug Improvement and Modernization Act of 2003 (MMA).

PhRMA is a voluntary, nonprofit association representing the country's leading research-based pharmaceutical and biotechnology companies, which are devoted to inventing and manufacturing medicines that allow patients to lead longer, healthier, and more productive lives. In 2003, PhRMA member companies invested an estimated $33 billion in discovering and developing new medicines. This research is seeking new cures and better treatments for many of the most serious diseases and conditions patients face, such as diabetes, heart disease, Alzheimer's, as well as many rare diseases and conditions. As leaders in the search for innovative new treatments that contribute to improved health care quality, effectiveness and efficiency, PhRMA is pleased to offer input on AHRQ research priorities in pursuit of this common goal.

Background:

PhRMA supports the development of high quality information about patients' medical treatment options. Empowering patients and physicians with high quality information will help ensure that our health system efficiently delivers the best possible results for all patients.

Section 1013 of the MMA creates a new program within the Agency for Healthcare Research and Quality for health outcomes research to improve the quality, effectiveness and efficiency of health care delivered under the Medicare, Medicaid, and State Children's Health Insurance (SCHIP) programs. The provision mandates a broad scope of research on health items and services, and directs HHS to follow an open public process for setting research priorities. PhRMA is submitting comments for setting research priorities that are consistent with the goals and broad research framework established by Congress under this section of the law.

In its notice published in the April 23 *Federal Register*, AHRQ announced the Department's process for setting research priorities under Section 1013, which includes creation of an inter-departmental HHS Steering Committee to review recommendations and make decisions on research priorities.

PhRMA appreciates the open public process HHS is establishing, as well as the HHS Steering Committee's consideration of our recommendations as it sets initial research priorities for AHRQ under Section 1013. We also appreciate the leading role AHRQ has taken since its inception in conducting health outcomes research aimed at improving health care quality and efficiency, and believe research under Section 1013 should build upon this work.

Section 1013 directs HHS to consider items and services from across the spectrum of care in setting priorities for outcomes research. It encourages HHS to consider items and services that may impose a high cost on the Medicare, Medicaid and SCHIP programs, that may be underutilized or overutilized, and that "may significantly improve the prevention, treatment, or cure of diseases and conditions (including chronic conditions) which impose high direct or indirect costs on patients and society."

Research shall address "the outcomes, comparative clinical effectiveness, and appropriateness of health care items and services (including prescription drugs)," and "strategies for improving the efficiency and effectiveness of" the Medicare, Medicaid and SCHIP programs, "including ways in which such items and services are organized, managed, and delivered under such programs."

Congress also mandated additional, longer-term goals for health outcomes research conducted under this section. The statute instructs HHS to develop, in collaboration with public and private entities, options for "provision of more timely information...regarding the outcomes and quality of patient care, including clinical and patient reported outcomes..."; "acceleration of the adoption of innovation and quality improvement..."; and development of management tools for the Medicare, Medicaid and SCHIP programs to improve oversight by State officials and support Federal and State initiatives to improve the quality, safety, and efficiency of services provided under these programs.

PhRMA is not making recommendations specific to development of options to achieve these longer-term goals in this document. However, we look forward to working with the HHS Steering Committee in this area in the coming months.

Establish an open, public process for setting research priorities and conducting and communicating research.

Section 1013 states that the Secretary "shall ensure that there is broad and ongoing consultation with relevant stakeholders in identifying the highest priorities for research, demonstrations, and evaluations to support and improve" the Medicare, Medicaid and SCHIP programs (§ 1013(a)(1)(C)(i)). It also says that activities shall be

implemented in a manner that "makes publicly available all scientific evidence relied upon and the methodologies employed (§ 1013(a)(3)(D)(i)).

PhRMA fully supports the initial steps HHS has taken to establish an open, public process for establishing research priorities under Section 1013 by publishing a notice soliciting recommendations for research priorities, establishing a Steering Committee to set research priorities, and scheduling a Town Hall meeting May 21st to solicit additional input.

In addition, consistent with the open, public process called for under Sec. 1013, we believe the Steering Committee should establish a record of work that makes open to the public all the input the Steering Committee has received on recommended research priorities – including input from agencies from which Steering Committee members are drawn – as well as the basis for the Steering Committee's decisions on research priorities under this Section. Finally, ongoing public input could be maximized by making public the Steering Committee's preliminary decisions for research priorities and allowing stakeholders to comment on them before they are finalized.

This approach would be consistent with the recommendations made by the Institute of Medicine in its 1992 report, *Setting Priorities for Health Technologies Assessment*. In this report, IOM said that AHRQ (then the Agency for Health Care Policy and Research) should use a method to set research priorities that is "explicit, so that people can trace backwards from results to inputs and so satisfy themselves that the process was fair."

Examine pharmaceuticals as part of a broader, patient-centered research agenda.

The April 23 *Federal Register* notice requesting recommendations for research priorities under Section 1013 includes a section on "Scope of Recommendations" which states: "While the statute does not limit the scope of the initial priority list, recent congressional activity suggests that the initial priority list should be directed toward evaluating existing evidence regarding the comparative clinical effectiveness of prescription drugs in anticipation of the Medicare prescription drug benefit."

PhRMA is concerned that, by limiting initial research priorities to prescription drugs, and limiting that research to one type of analysis – comparative effectiveness – HHS has made a significant priority-setting decisions outside of the public process mandated by 1013.

We recognize that, because pharmaceuticals are playing an increasingly important role in effectively, efficiently treating patients with a broad range of diseases and conditions, the research agenda under Section 1013 should and will include research on pharmaceuticals. Further, the scope of research under this section appropriately includes comparative effectiveness research on pharmaceuticals and other health care items and services.

However, we believe that research limited only to prescription medicines and only to comparative effectiveness analysis is neither an effective nor patient-centered approach to achieving the goal of improved quality, effectiveness and efficiency. Such an agenda would overlook (1) patients broader needs and priority issues for our health care system (2) IOM's 1992 recommendations to AHRQ for setting research priorities, and (3) the clear language of the statute passed by Congress just six months ago.

A research agenda that truly pursues better health care as the ultimate goal will take a broader view of the health care system, patient and physician needs, and the role of medicines within the health system. Prescription medicines account for just 10.5% of total health care spending, and can reduce spending on other more expensive interventions.[1] A narrow research agenda comparing medicines will do far less than the broader agenda described in Section 1013 to address our health system's fundamental problems related to health care quality, effectiveness and efficiency.

Such an agenda ignores a wide range of research envisioned under Section 1013, including research on: types of care other than pharmaceuticals; the effectiveness of items and services in improving health outcomes and reducing burden on patients and society; overuse, underuse and misuse of a range of health interventions; and strategies for improving the efficiency and effectiveness of care by improving the organization, management and delivery of these items and services.

A narrow research agenda also is wholly inconsistent with the IOM's 1992 recommendations to AHRQ, which clearly state: "priority setting [for technology assessment] should address clinical conditions"; and "technology assessments should compare the alternative technologies for managing a clinical condition."

Objective criteria like those established by the IOM in 1992 should drive the research priority-setting process under Section 1013.

The statute itself envisions a broad research agenda, stating that all research, evidence, and communication activities conducted under section 1013 "shall reflect the principle that clinicians and patients should have the best available evidence upon which to make choices in health care items and services, in providers, and in health care delivery systems." § 1013(b)(2) (emphasis added). This broad statutory language supports AHRQ's role of examining all facets of health care delivery in an integrated and holistic fashion.

Findings in the *Dartmouth Atlas of Health* underscore the importance of examining the full range of items and services available to treat priority diseases. The report states: "Under the rules of everyday medical practice, however, most non-drug

[1] See, e.g., Frank R. Lichtenberg, "Benefits and Costs of Newer Drugs: An Update," (Cambridge, MA: National Bureau of Economic Research, June 2002); PA Cowper, *et al.*, "Economic Effects of Beta-Blocker Therapy in Patients with Heart Failure," *The American Journal of Medicine*, 116 (2004): 2, 104-111, which found, "Beta-blocker therapy improves the clinical outcomes of heart failure patients and is cost saving to society and Medicare."

innovations escape rigorous evaluation...Failure to evaluate the outcomes of care" for such non-drug innovations is "an incredible waste of the opportunity to learn what works and what patients want."[2]

We appreciate HHS' recognition in the notice that the overall mandate of Section 1013 is for research on a broad range of health care items and services, and its indication that broader research priorities will be considered in future years. We believe this points to the importance of also setting a broader research agenda in the initial priority list, and the need for HHS to protect the integrity of an open, transparent process for setting research priorities. We look forward to providing additional input to the Steering Committee in the months ahead on this longer-term research agenda.

Set research priorities under Sec. 1013 consistent with Institute of Medicine recommendations and ongoing HHS work to improve health care quality.

The HHS Steering Committee should set priorities for outcomes research under Sec. 1013 based on diseases and conditions that place a heavy burden on patients, society, and health care finances, and examine the range of items and services available to patients for these diseases. This approach is essential to establishing a research agenda for AHRQ that meets patient needs and the goals of improving quality, effectiveness and efficiency of health care.

As a "problem solving" agency whose mission is improving health care quality, AHRQ can play an important role in helping policy-makers understand and address the fundamental challenges we face in seeking to improve health care quality, effectiveness and efficiency.

We note that a patient-centered approach (as well as the initial steps taken by HHS to set a public process for setting research priorities) is consistent with the IOM report *Setting Priorities for Health Technologies Assessment.*[3] This important report was mandated by Congress when it established the Agency for Health Care Policy and Research (renamed AHRQ). PhRMA believes the process developed and recommended by IOM provides a valuable benchmark and highly recommends that the HHS Steering Committee review this seminal work.

The Guiding Principles established in the IOM report say that the research priority-setting process must:

1. "Reflect [AHRQ's] mission which includes the potential to a) improve health outcomes; b) reduce inappropriate expenditures; c) redress inequity; and d) inform special social issues."

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[2] The Dartmouth Atlas of Health Care 1999, Center for Evaluative Clinical Sciences, Dartmouth Medical School, accessed April 24, 2004 at http://www.dartmouthatlas.org/99US/chap_7_sec_7.php.

[3] Institute of Medicine, Setting Priorities for Health Technologies Assessment, Molla S. Donaldson and Harold C. Sox, eds. Washington, D.C.: National Academy Press, 1992.

2. "Consider the needs of the users of the assessments which means, in general, to focus on *specific clinical conditions* and on *alternative approaches* to manage those conditions" (emphasis IOM's).
3. "Be efficient...by taking advantage of available data or, where data are lacking, of subjective judgments, rather than require the collection of new data."
4. "Be sensitive to its political context; [i.e.] be (and appear to be) objective, open, fair; invite input from a variety of interested parties; and present the logic of the process clearly and carefully to others".

The IOM report further recommended and defined seven priority-setting criteria and explained how to assign scores for each of them. These criteria include: disease prevalence, cost of intervention, clinical practice variations, burden of illness, likelihood an assessment will improve outcomes, potential that the results of an assessment will change costs and ethical, legal, social issues. Finally, the IOM report listed seven recommendations consistent with the guiding principles noted above.

By setting research priorities based on priority diseases and conditions, examining the range of health interventions for these diseases and conditions, and broadly examining the direct and indirect impacts of these interventions, the Steering Committee can implement a program that not only reflects the IOM recommendations, but complements the considerable work that AHRQ and other federal agencies have initiated over the past several years to address previously identified gaps in health care quality.

For example, the nation's first National Healthcare Quality Report (NHQR), released by AHRQ in December 2003,[4] is "built on measures focusing on the Nation's health care priorities as determined by associated morbidity, mortality, and opportunity for improvement." The priority diseases and conditions examined in the NHQR include cancer, diabetes, end stage renal disease, heart disease, HIV/AIDS, maternal and child health, mental health, respiratory disease, and nursing home and home health care.

The NHQR followed two seminal reports from the IOM – *Crossing the Quality Chasm: A New Health System for the 20th Century*[5], and *To Err Is Human: Building a Safer Health System.*[6] In its report on *Crossing the Quality Chasm*, the IoM Committee on Quality of Health Care in America recommended that, to begin the process of making fundamental quality improvements, AHRQ should identify "not fewer than 15 priority conditions" on which to focus efforts.

The IOM Committee on Quality explained: "Identifying priority conditions represents a starting point to support the organization of care, bring the evidence base

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[4] *National Healthcare Quality Report*, December 2003, Agency for Healthcare Research and Quality, Rockville, MD, AHRQ Publication No. 04-RG003

[5] *Crossing the Quality Chasm: A New Health System for the 21st Century*, Institute of Medicine, Washington, D.C.: National Academy Press, 2001

[6] Institute of Medicine, *To Err Is Human: Building a Safer Health System.* Linda T. Kohn, Janet M. Corrigan, and Molla S. Donaldson, eds., Washington, D.C.: National Academy Press, 2000

into practice, develop information technology and infrastructure to support care, and develop mechanisms to measure and pay for quality care." It added that "the system should be designed to optimize care for patients' needs across the entire continuum of care in the most effective and efficient way possible." A broad, disease-centered research agenda under Section 1013 would be consistent with these recommendations.

The Medicare, Medicaid and SCHIP programs and, more importantly, the patients who benefit from these programs, would be best-served by outcomes research priorities that build on existing efforts to improve quality and are centered on priority diseases and conditions.

Disease-specific research recommendations:

The HHS Steering Committee could make best use of resources under Section 1013 by targeting diseases and conditions that impose a significant burden on the Medicare and Medicaid programs and society overall. The charts below identify the 12 most expensive diseases for the nation and the Medicare and Medicaid programs. These figures are based on 2000 data from the AHRQ Medical Expenditure Panel Survey (MEPS).

12 Most Costly Diseases and Conditions for Medicare, Medicaid and Total U.S. Health Expenditures (dollars in billions)		
Medicare	**Medicaid**	**National Health Expenditures**
Heart conditions ($22.3)	COPD, asthma ($10.5)	Heart Conditions ($56.7)
Cancer ($12.6)	Mental disorders ($8.7)	Traumatic injury ($42)
Cerebrovascular disease ($9.9)	Normal birth/live born ($4.3)	Cancer ($38.9)
Trauma-related disorders ($9.2)	Heart conditions ($4.1)	COPD, asthma ($36.5)
Other circulatory conditions ($8.1)	Paralysis ($3.0)	Mental disorders ($34.4)
Pneumonia ($7.4)	Cancer ($2.9)	Hypertension ($23.4)
COPD, asthma ($6.8)	Infectious diseases ($2.9)	Normal birth/live born ($21.3)
Osteoarthritis and other non-trauma joint disorders ($5.8)	Hypertension ($2.7)	Other circulatory conditions ($19.5)
Diabetes mellitus ($5.2)	Perinatal Conditions ($2.5)	Diabetes mellitus ($18.3)
Hypertension ($4.9)	Trauma-related disorders ($2.4)	Osteoarthritis and other non-traumatic joint disorders ($17.7)
Kidney disease ($4.2)	Osteoarthritis and other non-traumatic joint disorders ($2.0)	Back problems ($17.5)
Mental disorders ($3.7)	Diabetes mellitus ($2.0)	Cerebrovascular disease ($14.9)

Many of these diseases are among those targeted by the Institute of Medicine in its 2003 report *Priority Areas for National Action: Transforming Healthcare Quality.* The goal of this report was "to identify priority areas that presented the greatest opportunity to narrow the gap between what the health care system is routinely doing now and what we know to be best medical practice," and used the criteria of "impact, improvability, and inclusiveness" in selecting priorities. Priorities selected in the report included asthma, cancer screening, pain control in advanced cancer, diabetes, obesity, hypertension, ischemic heart disease, major depression, severe and persistent mental illness, and stroke.[7]

Many of these diseases also are among those that impose the greatest health burden on society. A report in the Feb. 7, 2001 *Journal of the American Medical Association* reported on those diseases that cause the greatest loss of disability-adjusted life years in the U.S. For both men and women, ischemic heart disease ranked as the leading cause of loss in DALYs. Other major causes for men included cancers of the lung, trachea and bronchus; HIV/AIDS; cerebrovascular disease; chronic obstructive pulmonary disease; unipolar major depression; and diabetes mellitus. These diseases also caused major loss of disability-adjusted life-years among women, as did osteoarthritis and breast cancer.[8]

Based on these data, logical targets for initial research priorities under Sec. 1013 could include heart disease/hypertension, cancer, diabetes/obesity, and traumatic injury. The diseases we are recommending for priority research under Section 1013 have a major cross-cutting impact on both the Medicare and Medicaid programs and clearly have national public health significance as well. In addition, for most of the diseases we identify, appropriate use of pharmaceuticals plays an important role in the effective, efficient care of patients.

Several major studies recently have underscored that there is significant room for improving the quality of care received by patients who suffer from high-priority diseases and conditions. Notably, a narrow focus on comparative effectiveness of pharmaceuticals is absent from these studies identifying systemic quality issues in the United States.

A landmark study published in *The New England Journal of Medicine*, "The Quality of Health Care Delivered to Adults in the United States," found that a large percentage of patients with priority diseases and conditions are not receiving needed care. The study found that only 45 percent of patients with diabetes received the care they need; only 68 percent of patients with coronary artery disease received recommended

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[7] Institute of Medicine, *Priority Areas for National Action: Transforming Health Care Quality*, Karen Adams and Janet M. Corrigan, Eds., Washington, D.C.: National Academy Press, 2003

[8] CM Michaud, CJL Murray, BR Bloom, "Burden of Disease – Implications for Future Research," *JAMA* 285:535-539.

care; only 45 percent of heart attack patients received medications that could reduce their risk of death; only 54 percent of patients with colorectal cancer received recommended care, and less than 65 percent of patients with high blood pressure received recommended care. According to lead study author Elizabeth A. McGlynn, Ph.D., Associate Director of RAND Health, "Even people who had health insurance and access to health care services failed to receive some elements of good care."

An April 2004 report by the Alliance of Community Health Plans cites data showing that gaps in quality for just eight clinical measures result in over 57,000 avoidable deaths and $1.62 billion in avoidable costs each year. Most of the measures related to hypertension, diabetes, cancers, and cardiovascular disease. In addition, avoidable sick days for just five conditions totaled almost 41 million days in 2002.[9]

In addition, recent research comparing quality performance in 12 U.S. communities found that "quality performance ranged from 51% to 59%." The study, published in the May/June 2004 *Health Affairs,* also "found more variability in the quality of care for selected chronic conditions...both across conditions and across communities within the same condition. The article notes a number of potential strategies for improving quality at the community level that involve employers, health care systems, and community-based coalitions and outreach efforts.[10] AHRQ's contemplated focus for initial research under Sec. 1013 appears to largely overlook this series of findings.

This body of research illustrates why, in order to bridge existing gaps in quality, it is essential to take a comprehensive view of quality issues, rather than the narrow focus contemplated by HHS. Moreover, as Elizabeth McGlynn, Associate Director of RAND Health, states in a recent article in *Health Affairs*: "We do not know the net effects on health care spending of comprehensive strategies to improve quality. Nonetheless, if we want to improve quality and reduce costs, we should focus on quality improvement rather than cost reductions."[11]

By focusing on major diseases rather than individual technologies or services, HHS will maximize the opportunity to improve the health of beneficiaries and the quality and efficiency of care they receive. This is because health indices and efficiency are more related to access, care management, adherence to accepted standards of care and general health system delivery issues than they are to the selection of alternative therapies. This is why IOM made the recommendations it did in its 1992 report.

We will briefly describe the impact of just three diseases – heart disease, cancer, and diabetes – to illustrate the importance of research to help improve the quality,

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[9] A.J. Falk, JD, MPH, "Variations in Healthcare: Implications for Quality Improvement," Alliance of Community Health Plans, April 2004.

[10] E.A. Kerr *et al.,* "Profiling The Quality of Care in Twelve Communities: Results From The CQI Study," *Health Affairs,* Vol. 23, Issue 3, 247-256, May/June 2004.

[11] E.A. McGlynn, "There Is No Perfect Health System," *Health Affairs*, Vol 23, Issue 3, 100-102, May/June 2004.

effectiveness and efficiency of care delivered to Medicare and Medicaid patients with these and other high-priority diseases.

Heart Disease:

Heart disease is by far the most expensive, pervasive and deadly health issue facing the nation. Its impact is felt particularly by Medicare beneficiaries; it disproportionately affects African Americans (e.g., the prevalence and uncontrolled nature of hypertension), and it is a major cost-driver and health problem of Medicaid agencies.

In addition, a wide range of medical technologies, surgical procedures, diagnostic techniques and health delivery systems and services are available for heart disease, as well as a great deal of evidence that care is not optimal. For example, many patients who should be receiving antihypertensives and statin therapy under current guidelines are not being treated, and many treated patients are not fully compliant.[12]

While we believe the Steering Committee should target the overall disease category of "heart disease" rather than specific aspects of it in order to maximize efforts to improve outcomes, an examination of just one aspect of heart disease, hypertension, illustrates the importance of making this disease a priority for research. It also shows why a narrow focus on comparative effectiveness research alone will not help solve the basic challenges we face in improving health care quality and efficiency.

Despite the significant negative clinical and economic impact of hypertension, the disease "remains untreated or uncontrolled in near-epidemic numbers of Americans," Nash and Clarke report.[13] Almost half of the 43 million American adults with hypertension are not receiving necessary blood pressure medication, and for another 12 million who are being treated, the condition is inadequately controlled, according to the IOM report *Priority Areas for National Action: Transforming Health Care Quality.*[14]

Medicare-age patients (those 65 and older) make up the largest population of hypertensives, and the largest number for whom their blood pressure is not being treated or is inadequately controlled. The IOM report on priority areas finds that 8.1 million seniors with hypertension are untreated, with most of these seniors unaware of their condition, though 2.3 million are aware and untreated. An additional 3.5 million patients age 65 years or older have their hypertension treated and controlled.

Similarly, research shows that we are not making optimal use of effective treatments for heart disease. In fact, the 2002 *Economic Report of the President* cites research showing "many Americans who could benefit from the newly developed

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[12] Institute of Medicine, *Priority Areas for National Action: Transforming Health Care Quality*, Karen Adams and Janet M. Corrigan, Eds., Washington, D.C.: National Academy Press, 2003

[13] D.B. Nash and J.L. Clarke, "Putting ALLHAT into Perspective," *P&T* 28(9):585-590.

[14] Institute of Medicine, *Priority Areas for National Action: Transforming Health Care Quality*, Karen Adams and Janet M. Corrigan, Eds., Washington, D.C.: National Academy Press, 2003

cholesterol-lowering drugs do not receive them. Indeed, failure to use effective treatments has been estimated to result in 18,000 avoidable early deaths among heart attack patients in a year."[15]

Treatment of patients with acute coronary syndrome offers another illustration of the importance of broad research on heart disease. Acute coronary syndromes (ACS) are a major cause of morbidity and mortality in the United States. Evidence-based guidelines exist for patients with ACS, yet many patients in specific subpopulations are not receiving recommended care.[16] Further, higher levels of obesity and diabetes and an aging population likely will lead to an increase in the number of Americans suffering from acute coronary syndromes.

Research under Section 1013 should examine this and other aspects of heart diseases to provide information on the levels at which patients are receiving recommended diagnostic and therapeutic interventions, as well as potential steps to improve their appropriate use. This would improve the quality and efficiency of care delivered to these patients.

Cancer:

Cancer is the second leading cause of death in the U.S., after heart disease. Cancer caused 553,768 deaths in the U.S. in 2001, according to the American Cancer Society, accounted for 22.9% of all deaths in the U.S.[17]

A growing array of technologies to screen for, diagnose, and treat cancer has emerged in recent years. Declining mortality rates for cancer show that these advances are saving lives.

Regular screening examinations by a health care professional can result in the detection of cancers of the breast, colon, rectum, cervix, prostate, oral cavity, and skin at earlier stages when treatment is more likely to be successful. Cancers that can be detected earlier by screening account for about half of all new cancer cases. The 5-year relative survival rate for these cancers is about 84%. If all of these cancers were diagnosed at a localized stage through regular cancer screenings, 5-year survival would increase to about 95%.[18]

The American Cancer Society states that evidence suggests that about one-third of the 563,700 cancer deaths expected to occur in 2004 will be related to nutrition, physical

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[15] G.W. Bush, *Economic Report of the President* (Washington, D.C.: GPO, 2002), 180

[16] Braunwald E, Antman EM, Beasly JW, et al. ACC/AHA guideline update for the management of patients with unstable angina and non-ST-segment elevation myocardial infarction: A report of the American College of Cardiology/American Heart Association Task Force on Practice Guidelines (Committee on Management of Patients with Unstable Angina). J Am Coll Cardiol 2002;40:1366-1374.

[17] American Cancer Society, "Cancer Statistics 2004" accessed April 22, 2004 at http://www.cancer.org/docroot/pro/content/pro_1_1_Cancer_Statistics_2004_presentation.asp

[18] *Ibid*

inactivity, overweight or obesity, and other lifestyle factors, and thus could also be prevented.

Current evidence shows variation in rates of screening based on the type of cancer and geographic location. While the majority of women report being screened for breast and cervical cancer, less than half of Americans over the age of 50 have been screened for colorectal cancer. In addition, "there is considerable variation across the states in cancer screening. Improvement is possible and necessary," the NHQR says. The report adds: "More research is needed to understand why many people are diagnosed at an advanced stage of cancer."

In an IOM report ("Fulfilling the Potential of Cancer and Prevention and Early Detection"), the National Cancer Board concludes that to save the most lives from cancer, the health care community should be concentrating their resources on helping people to stop smoking, maintain a healthy weight and diet, exercise regularly, keep alcohol consumption at low to moderate levels, and get screened for breast, cervical, and colorectal cancer.

Focusing research priorities on a wider perspective has far more potential to save many lives and promote high-quality, efficient health care than does a narrow research focus.

Diabetes:

According to the American Diabetes Association (ADA), 18.2 million individuals, or 6.3% of people in the United States are estimated to have diabetes mellitus, which shortens life expectancy by up to 15 years. Because the onset of the disease occurs on average 4-7 years before diagnosis, many patients with diabetes go undiagnosed.[19]

Diabetes profoundly affects quality of life and represents a life-long burden on a patient's social support system; health-care access and utilization are therefore critically important. Since 1992, the direct medical cost of diabetes care has more than doubled – from $45.2 billion in 1992 to $91.8 billion in 2002. In addition, indirect expenditures resulting from lost work days, restricted activity days, mortality and permanent disability totaled $39.8 billion in 2002.

Among seniors over age 60, nearly one in five has diabetes. In 2000, diabetes contributed to the deaths of 213,062 people in the U.S.[20] According to the National Center for Health Statistics, diabetes mellitus ranked as the 6th leading cause of death in the United States in 2001, accounting for 71,372 deaths during that year.

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[19] American Diabetes Association: "All About Diabetes". Accessed April 30, 2004 at http://www.diabetes.org/about-diabetes.jsp

[20] Centers for Disease Control and Prevention, "National Diabetes Fact Sheet," (United States: CDC, November 2003), <http://www.cdc.gov/diabetes/pubs/pdf/ndfs_2003.pdf> (5 February, 2004).

An April 2004 study by Pharmetrics found that between 17 and 30 percent of insured people with diabetes are not treated with any anti-diabetic medicines or insulin over the course of an entire year.[21] It also found that patients who were entirely untreated with medicines had higher diabetes-related health costs and more hospitalizations than patients who had at least some treatment with medicines during the year. All patients included in the study had health insurance, including insurance coverage for prescription drugs.

The study also found that patients who adhered most closely to a prescribed drug regimen for the treatment of diabetes had a nearly 40 percent lower chance of being hospitalized during the year than the patients who had the lowest adherence (10 percent versus 16 percent). The roughly $300 cost per patient of increased medicine usage among the patients with the highest level of adherence was more than offset by $610 per patient savings on diabetes-related hospital care.

To meet the growing public health threat posed by diabetes, more research is needed to identify the most effective, efficient approaches to ensure that people with diabetes are diagnosed and are controlling their blood glucose levels.

Additional recommendations for disease-specific research:

Research conducted under Section 1013 should help ensure that patients and physicians have the information to make the best use possible of health care items and services that can reduce the burden of priority diseases and conditions. To that end, PhRMA recommends that research under Section 1013 include:

1) Analysis of the extent to which the health care system is making optimal use of interventions known to work. Identify areas where interventions are being overused, underused, or misused, and the clinical impact of these sub-optimal uses, and identify potential strategies for making better use of interventions known to be effective and avoiding uses of interventions known to be ineffective or unsafe. Several potential aspects of this research have been described in these comments.

2) Examination of issues related to patient therapy adherence and persistence behavior. Most medicines provide therapy for a given period of time that is dependent upon the patient for effective, continuous delivery. When patients do not comply with prescribed therapy, it can create as significant a barrier to quality as the failure to prescribe it in the first place. When examining effectiveness of medicines in treating priority diseases, it is important to consider the extent and causes of, and potential solutions to, problems related to therapy compliance.

3) Assess the broad impact of priority diseases and conditions beyond their effect on government health spending. Research under Section 1013 should examine effects

[21] "Examination of Treatment Patterns and Effects of Medication Taking Behaviors among Patients with Diabetes," Pharmetrics, Inc. April 21, 2004

such as the increased demand these diseases create for other social services, loss of taxable income and workdays.

4) Identify and evaluate key policy considerations in implementation of the Medicare Modernization Act to improve quality, effectiveness, efficiency of care provided for beneficiaries with selected diseases and conditions. These could include issues such as Medicare quality initiatives, cost-sharing and other incentives that might encourage or discourage provision of quality care, as well as disease management demonstration opportunities.

5) Study and evaluate effective communication to Medicare and Medicaid beneficiaries to support physician-patient decision-making. Decisions about whether to initiate a treatment, and which therapies to choose, are complex, multi-factorial choices that must be made between a patient and his or her physician. Making these decisions requires the patient and caregiver to consider issues such as concomitant therapies, comorbidities, drug safety profiles, and other issues related to the individual's medical needs and preferences. It is critical to provide information about treatment choices in ways that recognize these issues and communicate them in ways that the patient can understand.

Conclusion:

The research program mandated by Congress under Section 1013 of the MMA offers a valuable opportunity for the HHS Steering Committee and AHRQ to pursue health outcomes research, demonstrations and evaluations that improve the quality, effectiveness and efficiency of health care received by patients under the Medicare, Medicaid and SCHIP programs.

The framework established by Congress for conduct of this research will help achieve this goal. Key aspects of the framework include a broad health system scope of research; an emphasis on diseases and conditions with a significant impact on patients and society and on interventions that may be subject to over- or underuse; the centrality of the patient and caregiver in making treatment decisions; examination of the broad range of health system issues that play a role in improving health care quality and efficiency; and the importance of effective communication with patients and caregivers.

In making decisions on research priorities under Section 1013, we recommended that the Steering Committee follow the recommendations made by IOM in its 1992 report on setting research priorities. These recommendations are consistent with the framework established under Section 1013 and support the goals of improved quality, effectiveness and efficiency.

The research projects PhRMA recommends follow the disease-centered approach endorsed by IOM, and will help promote the optimal use of pharmaceuticals and the many other health care items and services important to patients. We look forward to working with the HHS Steering Committee and AHRQ in the coming months as you establish research priorities under Section 1013 of the Medicare Modernization Act.

Sincerely,

Richard I. Smith